## (12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

## (19) World Intellectual Property Organization International Bureau





(43) International Publication Date 25 August 2005 (25.08.2005)

**PCT** 

## (10) International Publication Number WO 2005/077093 A2

(51) International Patent Classification:

\_\_\_\_

Not classified

(21) International Application Number:

PCT/US2005/004345

(22) International Filing Date: 7 February 2005 (07.02.2005)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data: 60/542,586

6 February 2004 (06.02.2004) US

- (71) Applicant (for all designated States except US): BIOMARIN PHARMACEUTICAL INC. [US/US]; 105 Digital Drive, Novato, CA 94949 (US).
- (72) Inventors; and
- (75) Inventors/Applicants (for US only): ZANKEL, Todd [US/US]; 46 Galli Drive, Novato, CA 94949 (US). KAKKIS, Emil, D. [US/US]; 2512 Laguna Vista Drive, Novato, CA 94949 (US).
- (74) Agent: McMILLIAN, Nabeela, R.; Marshall, Gerstein & Borun LLP, 233 South Wacker Drive, 6300 Sears Tower, Chicago, IL 60606-6357 (US).

- (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IS, IT, LT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GO, GW, ML, MR, NE, SN, TD, TG).

## Published:

 without international search report and to be republished upon receipt of that report

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: MANUFACTURE OF HIGHLY PHOSPHORYLATED LYSOSOMAL ENZYMES AND USES THEREOF

(57) Abstract: This invention provides compositions of highly phosphorylated lysosomal enzymes, their pharmaceutical compositions, methods of producing and purifying such compounds and compositions and their use in the diagnosis, prophylaxis, or treatment of diseases and conditions, including particularly lysosomal storage diseases.